



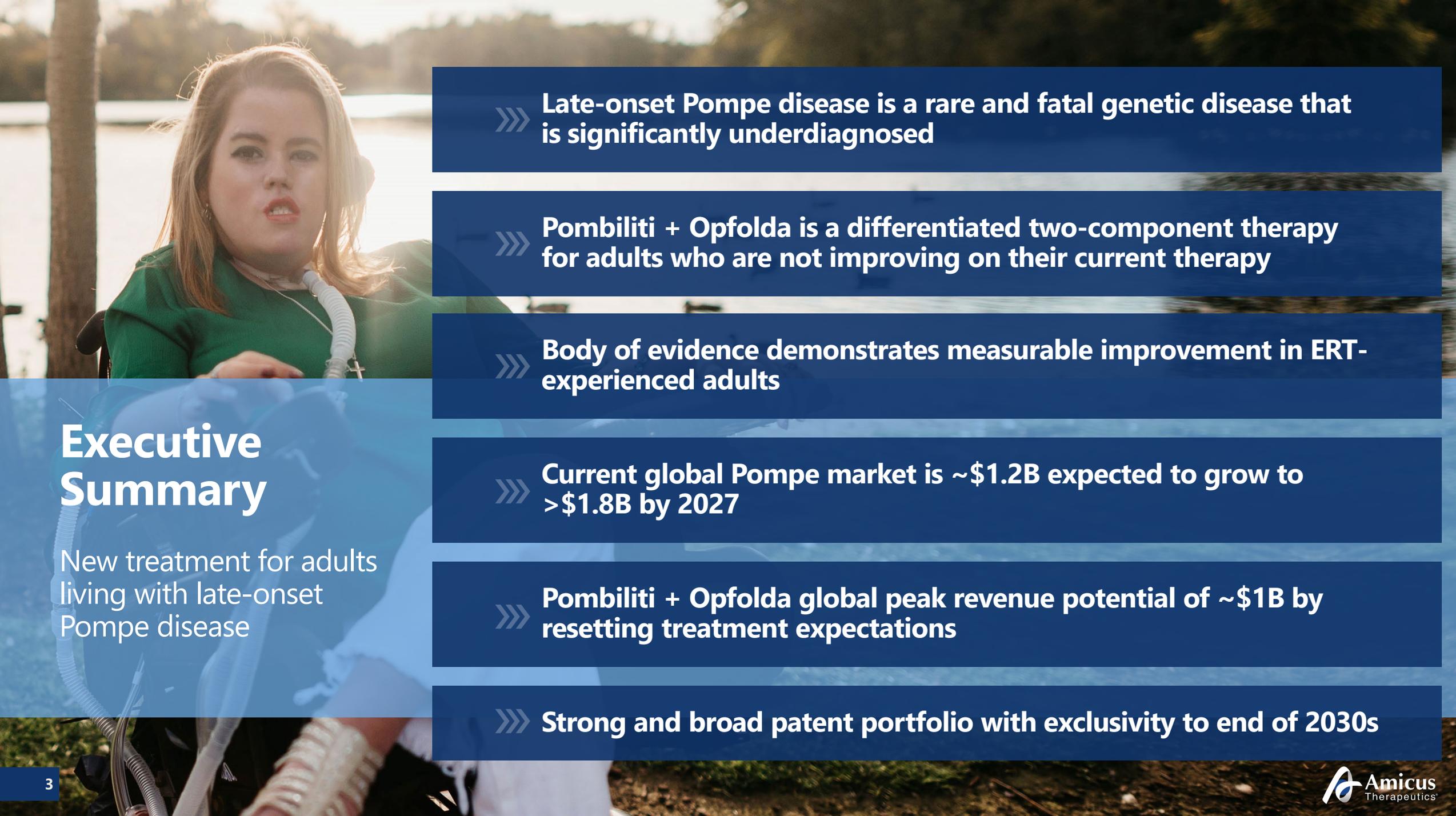
**U.S. Approval Call:  
Pombiliti™ (cipaglucosidase alfa-atga) +  
Opfolda™ (miglustat)**

September 28, 2023



# Forward-Looking Statements

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to approval and commercialization plans for Pombiliti and Opfolda in the United States. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that we may not be successful in commercializing Pombiliti + Opfolda in the United States, the potential that public and commercial payors will not reimburse Pombiliti + Opfolda, the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete all of our commercialization and manufacturing activities. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2022, as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, filed the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*



## Executive Summary

New treatment for adults living with late-onset Pompe disease

»» Late-onset Pompe disease is a rare and fatal genetic disease that is significantly underdiagnosed

»» Pombiliti + Opfolda is a differentiated two-component therapy for adults who are not improving on their current therapy

»» Body of evidence demonstrates measurable improvement in ERT-experienced adults

»» Current global Pompe market is ~\$1.2B expected to grow to >\$1.8B by 2027

»» Pombiliti + Opfolda global peak revenue potential of ~\$1B by resetting treatment expectations

»» Strong and broad patent portfolio with exclusivity to end of 2030s

# Late-onset Pompe Disease (LOPD) Overview

Late-onset Pompe disease is a rare, debilitating, and life-threatening lysosomal disorder caused by a deficiency of the enzyme acid alpha-glucosidase (GAA)



~5,000-10,000 people diagnosed globally; Significant underdiagnosis

Diagnosed at different stages of life, from childhood to adulthood

Majority of patients on current standard of care decline after ~2 years

Respiratory failure and loss of motor function are major causes of morbidity and mortality

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

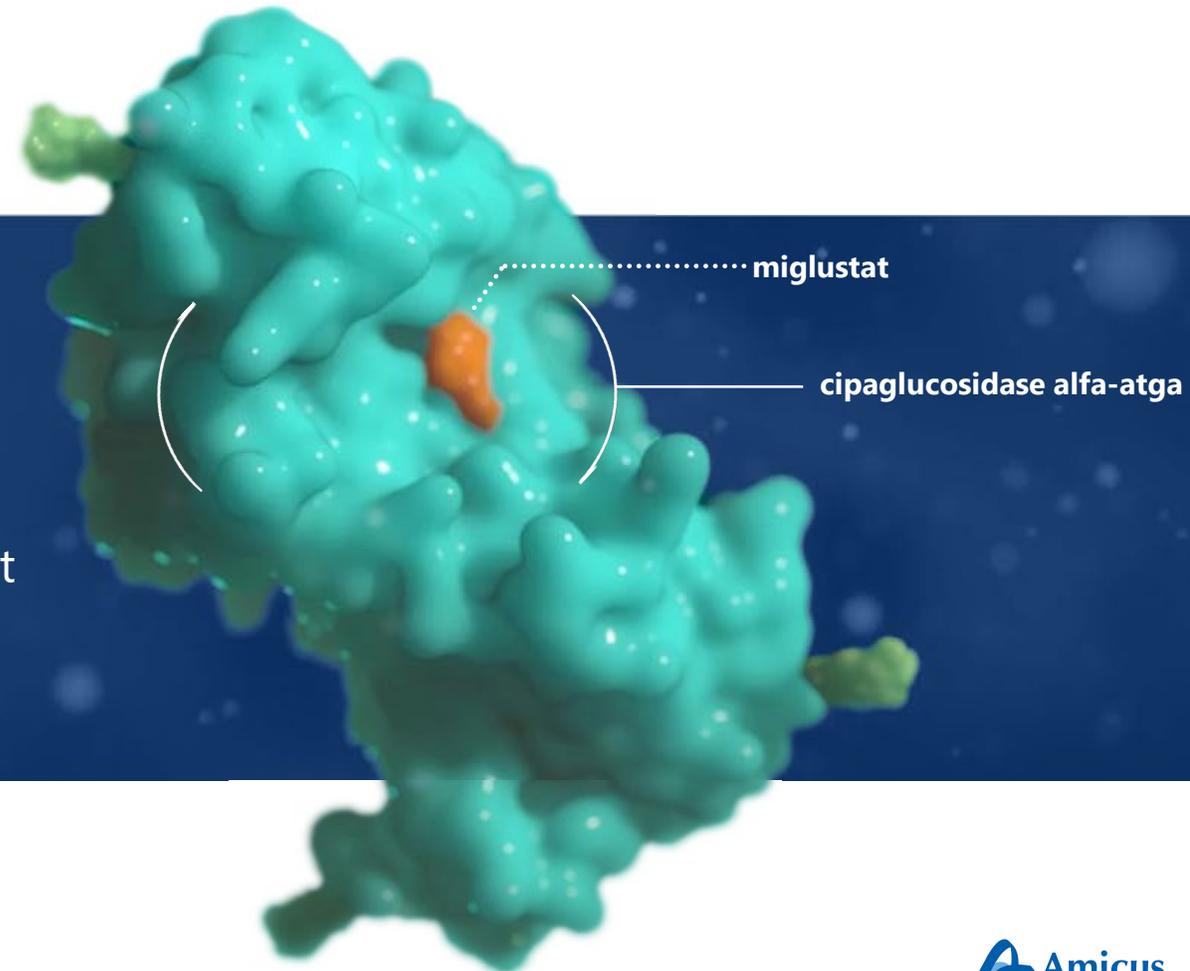
Symptoms include progressive muscle weakness, particularly skeletal and respiratory muscles, that worsens over time

~\$1.2B+ global Pompe ERT sales<sup>1</sup>

# Pombiliti + Opfolda: An Innovative Approach to Pompe Disease

Our scientists created a uniquely glycosylated and highly phosphorylated ERT that enhances targeting to key affected muscles and is co-administered with an enzyme stabilizer

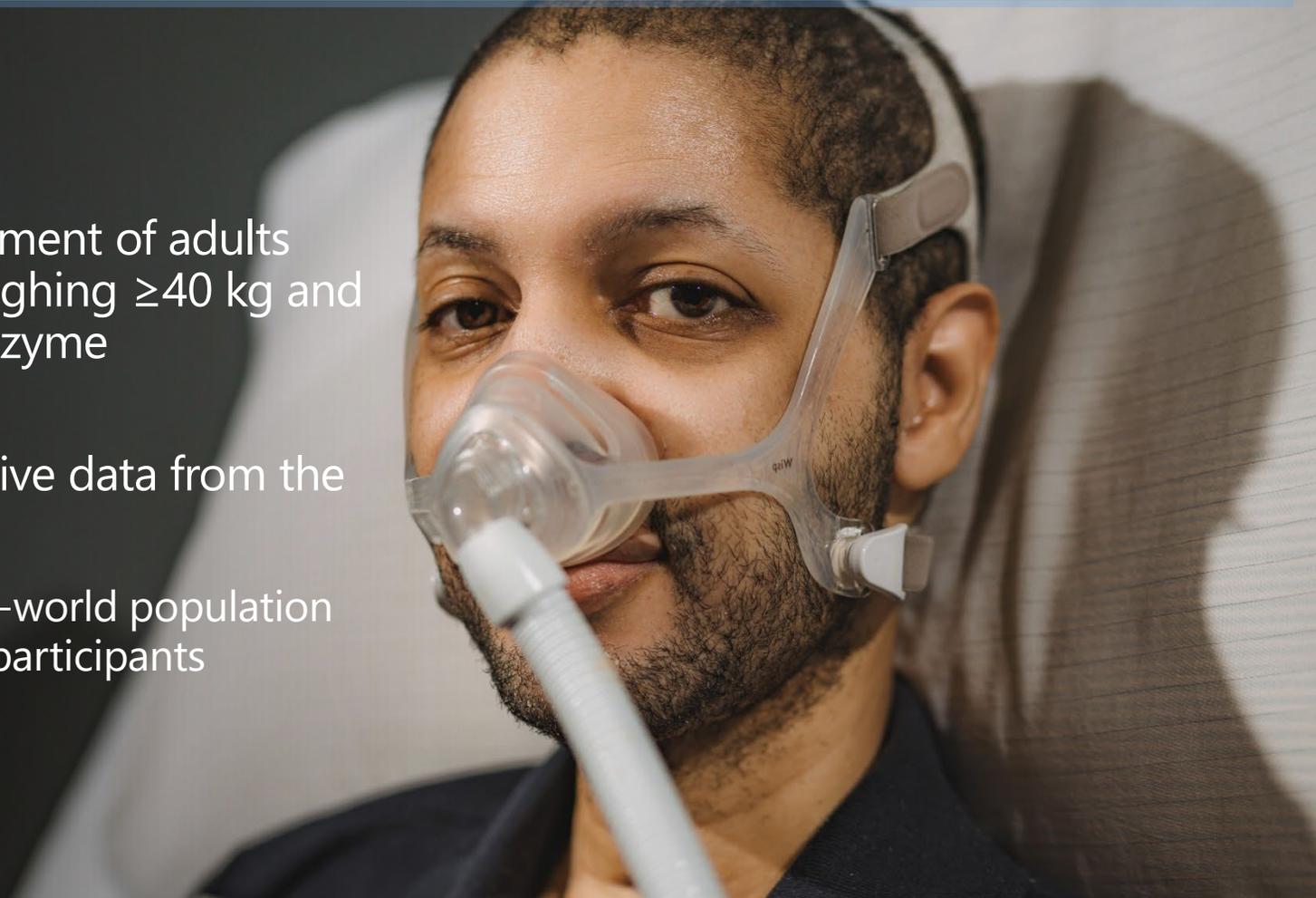
- Pombiliti + Opfolda is a two-component therapy combining cipaglusosidase alfa-atga, an ERT, with miglustat, an orally administered enzyme stabilizer
- Pombiliti is expressed in a unique cell line producing a naturally glycosylated and highly phosphorylated enzyme that can be properly processed to its mature form, which are both required for greater lysosomal GAA activity<sup>1</sup>



# U.S. Label Highlights

## Pombiliti + Opfolda approved in the United States

- Pombiliti + Opfolda indicated for the treatment of adults with late-onset Pompe (LOPD) disease weighing  $\geq 40$  kg and who are not improving on their current enzyme replacement therapy
- This indication is approved based on positive data from the Phase 3 pivotal study (PROPEL):
  - The only controlled trial to study the real-world population of both ERT-naïve and ERT-experienced participants

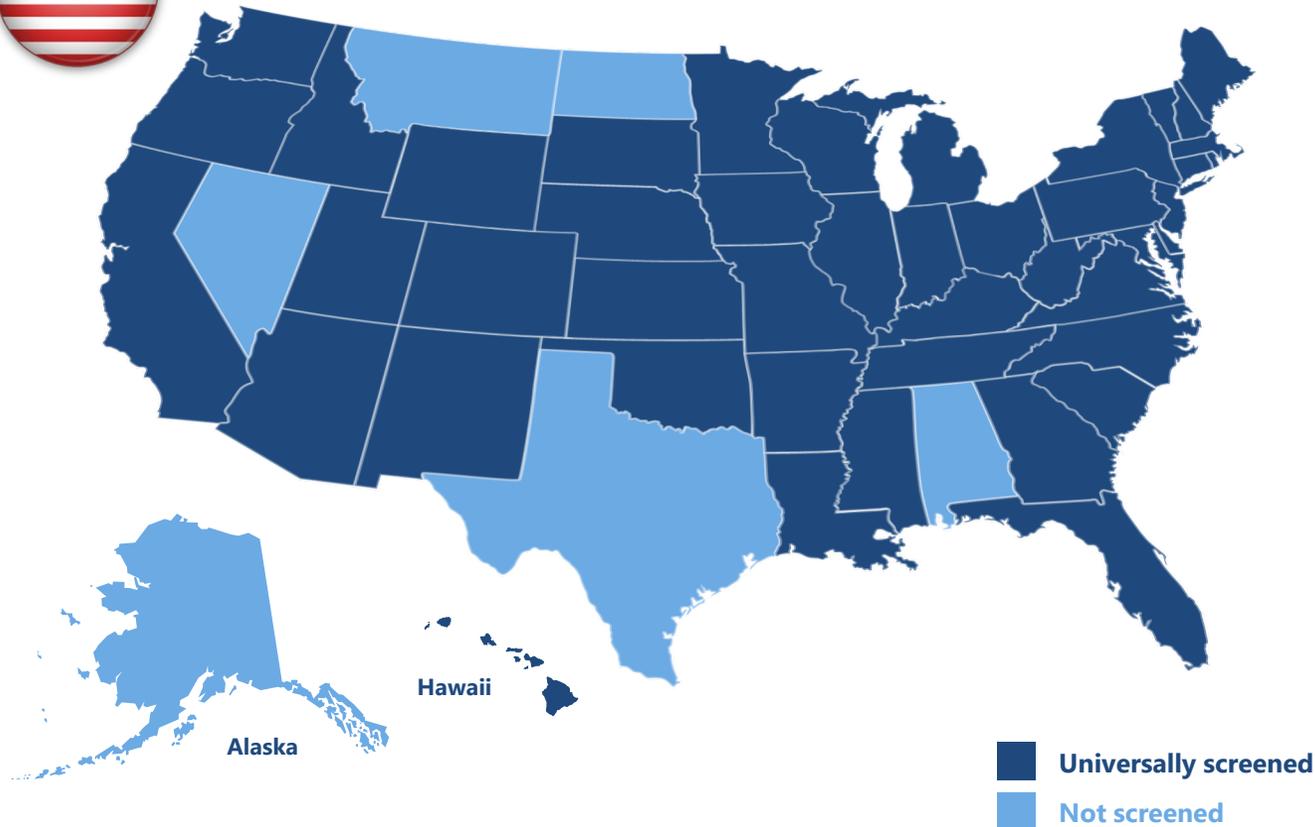


# Pombiliti + Opfolda U.S. Opportunity

U.S. Pompe market currently represents a sizeable market opportunity of >\$500M<sup>1</sup>



## Pompe Disease Newborn Screening in 44 U.S. States<sup>2</sup>



- ~80% of the 800 treated patients in the U.S. are adults<sup>1</sup>
- Currently ~40 patients in the U.S. on Pombiliti + Opfolda through clinical trials are eligible for commercial product and are expected to transition
- 88% of states screen for Pompe disease in newborns which continues to support diagnosis of Pompe disease

<sup>1</sup> Amicus Data on File from Market Mapping

<sup>2</sup> <https://www.newsteps.org/resources/data-visualizations/newborn-screening-status-all-disorders>

# Pombiliti + Opfolda Launch Activities

Experienced and passionate rare disease commercial and medical organizations poised for second successful launch



- Market mapping and account profiling completed
  - Sales team visiting top accounts in first 30 days
  - Promotional materials developed
- Product labeled and available for sale within 2 weeks
- Successful transition of all clinical trial patients by year-end
- Market Access: Payer ad boards and pre-approval information exchange meetings completed
- Patient hub: All case managers and patient education liaisons hired and trained

# Pricing Promise

“*Our medicines must be fairly priced and broadly accessible*”

- Amicus Founding Belief

## Pombiliti + Opfolda U.S. Pricing

- Combined price of Pombiliti + Opfolda slightly below current treatments
- **Pricing PROMISE:** Amicus will limit price increases to CPI-U (consumer price index)
- **Pledge for a Cure:** Amicus pledges to reinvest a portion of our profits into R&D of new treatments for Pompe disease until there is a cure
- Provide world-class patient support services, including needs-based financial support globally to ensure all who need our therapies have access to them

# 2023 Strategic Priorities

**1** Double-digit Galafold<sup>®</sup> revenue growth of 14-18% at CER<sup>1</sup>

**2** Secure FDA, EMA, and MHRA approvals for Pombiliti + Opfolda ✓

**3** Initiate successful global launches of Pombiliti + Opfolda ✓

**4** Advance best-in-class, next-generation Fabry and Pompe pipeline programs and capabilities

**5** Maintain strong financial position on path to profitability



**Thank You**

